Clinical Studies & You: Your Role, Your Choice

Adam Sherman
Research Director, IFOPA

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You Have a Choice
You Have the Power to:

- Address key questions about FOP
- Assess drug candidates
- Accelerate the discovery and assessment of FOP treatments

You Have the Power to be part of the solution!
Outline for our discussion

• What are the different clinical trial stages, and how does a drug get to market?

• What are the risks and benefits of participating in a clinical trial?

• How do I decide whether to participate in a clinical trial?

• Questions and discussion
The path to drug approval can be complex.
Drug Discovery and Development Pathway

Source: Pharmaceutical Research and Manufacturers of America
Types of Clinical Research

Impact of a disease on morbidity, mortality, healthcare resource utilization, health-related quality of life.

Treatment studies test new (or repurposed) drugs, new combinations of drugs, or procedures
- Evaluates safety and effectiveness of a drug or intervention
- There are risks

Natural history studies provide valuable information about how a disease and a person’s health progress
- No attempt to stop disease progression
- Limited risk
- Informs future clinical trials

Diagnostic/Biomarker
Clinical Trials
Screening
Quality of Life/Burden of Illness
Treatment
Registry
Natural History
Clinical Trial Concepts

- **Placebo (control)**: An inactive product that resembles the test product, but without a treatment value.

- **Randomization**: Assigning two or more alternative treatments by chance to demonstrate the new products therapeutic effectiveness.

- **Study Blinding**: Single or Double Blind – study participants do not know which medicine is being used so they can describe what happens without bias and preventing investigators from influencing results.

- **Cross-over**: Participants get both the placebo and experimental treatment but do not know the order.

- **Primary Endpoint**: The primary (or most important) question being asked by a trial.
Deciding to participate in a clinical trial is a personal decision and should take into consideration the **benefits** vs the **risks and costs** of participating.
Benefits & Risks of participating in clinical trials

**Possible benefits**
- If the new treatment or intervention is proven to work, patients may be among the first to benefit.
- Patients will receive, at a minimum, some of the best standard treatment.
- Patients have a chance to help and improve care for others.

**Possible Risks**
- The drug may not work (or may work for others, but not you).
- Drugs have side effects and the full extent of adverse effects are not yet known.
- You may not receive a drug at all (given a placebo).
How are you protected in a clinical trial?

1. Informed consent
2. Scientific Review
3. Institutional Review Board
4. Data Safety Monitoring Board
INFORMED CONSENT: a document that describes

- Why the research is being conducted
- What researchers want to accomplish
- What will be done during the trial and for how long
- What risks and discomforts are involved in the trial
- What benefits can be expected from the trial
- What other treatments are available
- You have the right to leave the trial at any time
- Confidentiality of information collected during the clinical trial

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Scientific Review
- Clinical studies include advice from experts in the field
- Designed by medical professionals with scientific expertise
- Documented through clinical protocols that have strict guidelines for study conduct
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**Institutional Review Board**
- Administrative body established to protect the rights and welfare of human research subjects
- Independent committee of physicians, statisticians, community advocates and others
- Every clinical trial in the U.S. must be approved and monitored by an IRB
- The IRB determines whether the risks involved in a study are reasonable with respect to the potential benefits
Data Safety Monitoring Board

Group of independent individuals with expertise to review data from a clinical trial to ensure:

- Risks are minimized
- Data integrity is maintained
- A trial is stopped if safety concerns arise or as soon as its objectives have been met

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Are you eligible to participate?

Inclusion & Exclusion Criteria

Every trial has eligibility criteria called inclusion and exclusion criteria.

- Criteria state what patient characteristics make the patient eligible or ineligible
- Not a personal rejection
- Used to identify appropriate participants and keep them safe
- Help ensure that researchers will be able to answer the questions they plan to study
- Common criteria include: age, disease stage, previous treatment, health and other coexisting medical conditions
Considerations for trial participation

- Potential safety and effectiveness of the medication
- Sponsorship and conduct of the trial
  - Access to treatment during the trial, after the trial (e.g., extension trials)
  - Route of administration
  - Dosing frequency
  - Travel to sites/time commitment
- Additional study procedures
- Trial costs
- Plans to publish the results
Questions Participants Can and Should Ask!

Some questions you might ask about the research include:
- What is the purpose of the study?
- Who is sponsoring the study?
- Who has reviewed and approved this study?
- Why does the research team think the treatment/drug will work?

Some questions about your participation in the study include:
- Where is the study site?
- What kinds of therapies, procedures, and/or tests will I have during the trial?
- Will they hurt? If so, for how long?
- How long will the study last?
- How often will I have to go to the study site?
- Who will provide my medical care after the study ends?
- Will I be able to take my regular medications during the trial? Do I need to avoid any medications?
- What medications, procedures, or treatments must I avoid while in the study?
- Will I have to be in the hospital during the study?
- Will I be able to find out the results of the trial?
- Will I have to pay anything to participate in the study?

Some questions about risks include:
- What is known about the potential adverse effects of the treatment?
- What are the possible immediate and long-term side effects?
Caution Using Social Media

**Don’t** post about your experience in the trial, including about side effects or how you think the drug is working

**Don’t** solicit trial advice online or from people other than the clinical coordinator or investigator at your trial site
Available resources

- Your treating physician and/or clinical investigator!
- [www.clinicaltrials.gov](http://www.clinicaltrials.gov)
- [http://www.ifopa.org/clinical_trials](http://www.ifopa.org/clinical_trials)
- [https://www.fda.gov/ForPatients/ClinicalTrials](https://www.fda.gov/ForPatients/ClinicalTrials)
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